Format: Abstract

J Pediatr Hematol Oncol. 2018 Oct 23. doi: 10.1097/MPH.0000000000001339. [Epub ahead of print]

Nivolumab in the Treatment of Recurrent or Refractory Pediatric Brain Tumors: A Single Institutional Experience.

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Successful use of immune checkpoint inhibitors in a variety of cancers has generated interest in using this approach in pediatric brain tumors. We performed a retrospective review of 10 consecutive children (6 boys, 4 girls; ages, 2 to 17 y), with recurrent or refractory pediatric brain tumors (5 high-grade glioma, 1 low-grade glioma, pineoblastoma, medulloblastoma, ependymoma, and CNS embryonal tumor, NOS) treated at Rady Children's Hospital San Diego from 2015 to 2017 with the immune checkpoint inhibitor nivolumab (3 mg/kg every 2 wk). Eight of 10 patients received prior chemotherapy and 9 radiation therapy. Nine patients had radiographic disease progression (median, 2.5 doses). Median time to progression was 5.5 weeks (1.6 to 24 wk). Three patients (2 with high-grade glioma, 1 with CNS embryonal tumor NOS) showed a partial response to treatment at the primary tumor site and 2 of 3 had progression of metastatic disease. Grade 2 toxicities were observed without dose limiting side effects. Tumor mutation burden (TMB) was low to intermediate (median, 1.3; range, 0 to 6.3). Median survival for PD-L1 positive patients was 13.7 weeks versus 4.2 weeks for PD-L1 negative patients ($\rho=0.08$) nivolumab was well tolerated in our series of pediatric recurrent brain tumors with some transient partial responses in patients with positive PD-L1 expression and higher TMB. Our findings suggest that the use of immune checkpoint inhibitors in pediatric brain tumor patients should be limited to those with elevated PD-L1 expression and TMB.

PMID: 30681550 DOI: 10.1097/MPH.0000000000001339